

Spring 2023

MNDnews

The newsletter of the Motor Neurone Disease Association of Tasmania



Warming our hearts
**Big Freeze
events across
Tasmania**

mnd 
Tasmania

Until there's a cure, there's care

MESSAGE FROM THE PRESIDENT

Welcome to the 2023 Spring edition of our newsletter. Spring brings renewal and warm weather, as well as the biggest event on our calendar – the Walk to D’Feet MND! We couldn’t be more excited about the Walk. We love catching up with our members, our community, and our amazing supporters! Please make sure to mark out the 15th of October on your calendar, and join us once again at the Queen’s Domain.

As always, we will have a fun-packed morning filled with activities and the usual great prizes up for grabs. It is also a great opportunity to meet and hear from some research leaders and PhD students from UTAS. Our website is booming with different teams’ fundraising for this great day! Please get in touch if you’d like to be involved.

During winter in Tasmania, we’ve had some very brave people participating in Big Freeze events across the state. From the pictures we saw, they seemed to have had so much fun, and we thank all involved in such great events. Our hearts are warm!

You will read a very thorough and interesting article on PEG tube feeding by Paul Kelly. It is a privilege to have Paul and his expertise in communication contributing to our newsletter. Thank you, Paul!

We are so grateful for all the support we receive from all of our members and from our amazing community. MND Tasmania’s main goal is to support and improve the quality of life for those in Tasmania who are living with MND, and their families. We couldn’t do it without you.

We welcome new members to the MND Tasmania family. We are here for you and will support you on the journey ahead. Please reach out to us through the Board and through our wonderful MND Advisors.

We love to hear from our members, so please write and share your stories, opinions, questions, or any thoughts about this newsletter. Write to me at info@mndatas.asn.au or see our contact details on the back page.

Kate Todd
President



PERSONAL STORY

Deciding on a PEG Tube: Options for Enteral Feeding

By Paul Kelly, currently living with MND

For patients with MND, difficulties with swallowing are often one of the most challenging aspects of the disease. As muscles in the mouth and throat weaken, eating and drinking become increasingly difficult. At some point, patients may need to consider alternative methods for getting nutrition and preventing choking or aspiration pneumonia.

One option is to receive nutrition through a percutaneous endoscopic gastrostomy (PEG) tube. A PEG tube is a flexible plastic tube that is inserted directly into the stomach through the abdominal wall, allowing liquid nutrition to be delivered without having to swallow. It bypasses the mouth and throat to provide nutrition and hydration. For many MND patients, a PEG tube can help maintain comfort and quality of life by ensuring adequate nutrition is received.

However, the decision about whether or not to get a PEG tube is a complex one that should involve careful consideration of the risks and benefits, as well as an individual's personal goals and priorities. It is important for patients and their families to understand all of the factors involved so that they can make the choice that is best for their unique situation.

Medical guidelines generally recommend considering a PEG tube insertion when a patient is no longer able to maintain their nutrition and hydration needs orally. Specifically, PEG tube placement may be advised when swallowing problems and inability to meet over 50% of daily caloric needs by mouth develop, when there is risk of choking, coughing, or aspiration during meals, and when dehydration is a concern.

Some potential advantages of receiving nutrition through a PEG tube include maintaining or improving nutritional status, reducing risk of aspiration pneumonia, and providing an easy, non-invasive method for giving hydration and medications through the tube.

At the same time, placing a PEG tube does carry some disadvantages and risks that patients should be aware of, including ongoing care required for the tube, no chewing or solid foods can be consumed orally while the tube is in place, and cosmetic concerns about the tube being externally visible on the abdomen. Obtaining a temporary or permanent abdominal tattoo at insertion site may help.

Because the factors involved are complex with both advantages and disadvantages to weigh up, personal concerns and individual preferences play a big role in this decision. Patients should reflect deeply on how much the ability to eat orally through natural swallowing is valued personally, and at what point does this outweigh risks of malnutrition/aspiration.

To determine the best choice for their situation, patients should discuss all aspects openly with their healthcare team and family members. The multidisciplinary MND clinic team can also connect patients to other MND patients who have experienced tube placement and those who have declined tubes to share their perspectives. Additionally, patients may consider spending time observing tube feedings in action to better understand what is involved. Trial runs with oral nutritional supplements or temporary nasogastric tube can also help assess tolerability before making a long-term commitment.

With thorough understanding of options, advice, and personal priorities examined, patients are empowered to select the choice they are most comfortable with. While not an easy decision, taking the time for careful consideration helps to align medical intervention choice to an individual's unique goals, values and definition of quality of life.

With open communication and inclusion of personal preferences in the decision-making process, MND patients can feel confident that they have thoroughly explored PEG tube placement, and have determined the best path forward for their own well-being and comfort as the disease progresses. The healthcare team remains available for ongoing support no matter which choice is made.

MND TASMANIA SAYS **THANK YOU**



Big Freeze at St Brendan-Shaw College

It was a perfect winter's day when students gathered around the St Brendan-Shaw College's oval at lunchtime to raise funds for MND Tasmania.

The 'Big Freeze' for MND Tasmania was a spectacular and colourful event; with students, staff, and SRC Executives heading down the slide into a freezing ice bath. Their goal of raising \$1,000 was easily surpassed, having reached a total of over \$2,000 in funds raised.

We thank everyone who organised, participated, and contributed to this amazing effort!



Big Freeze at Port Sorell

Chris Symonds, our Vice President & Public Officer, attended the Annual Port Sorell Primary School Big Freeze. Chris talked to students and teachers present about MND and how it affects a person's life, and he also talked about our work in Tasmania.

They have raised over \$1,300 in funds that will stay in Tasmania to support those living with MND and KD and their families, as well as equipment and research!

It was a fun day where students and teachers braved the cold for a great cause. Thank you all so much!

New Members

We welcome new members living with MND and their families to the MND Tasmania community, network, and services. Please feel free to contact our MND Advisors for Tasmania (see contact details on the back page). The MND Advisor service is managed on our behalf by MND Victoria, with MND Advisors who travel State-wide as required to support our members on a needs-basis.

Thank you for your support!

We are grateful for the support we have received from Andrew Bovill, Hagley Farm Primary School, Perth Baptist Church – Arts and Crafts, Jacob George, William and Penny Cromarty, Pamela Greenwood, Julia Greenhill, Stuart Heron, Margaret Taylor, Gill Anning AWC Pty Ltd, Tony Davies, Ken Fitzpatrick, Mel Gray, Rebecca Cracknell, Kellie Hills, Nicole Webster, Brent Webster, Andrew Pilgrim, Andrew Luttrell, Campania Fire Brigade, Jacob and Jessica Baxter.

We also acknowledge the ongoing support received from Margaret Eldridge.

We have received donations in memory of Peter Webster and Neitha Cracknell.



UPCOMING EVENTS

Walk to D'Feet MND this October

We are organising our upcoming Walk to D'Feet MND and we couldn't be more excited! It is always such a great day where we catch-up with our wonderful community and do a few laps around the Domain.

Get ready for an amazing morning of making memories, remembering our loved ones, and connecting with new and old friends.

All funds raised will stay in Tasmania to care and support those currently living with MND/KD. We are looking forward to seeing you there!



When: Sunday, 15th October 2023 |
10:00AM - 1:00PM

Where: Upper Domain Rd, Queens Domain,
Hobart, Tasmania

Registrations are now open on our website.
Visit www.mndatas.asn.au/events/6/walk-to-dfeet-mnd-2023 or scan the QR code below.



MND Support Groups

The MND Support Groups in Tasmania are independently run by community members for community members. We appreciate their willingness to devote their time and volunteer for such a worthy cause.

North West MND Support Group

The NWSG in Ulverstone invites all MND Tasmania members and supporters to its meetings.

When: At 11 am on the first Wednesday of the month, except January

Where: Ulverstone Returned Servicemen's Club Back Room, 21 King Edward Street, Ulverstone

Contact: Junene Stephens is the secretary and can be contacted on 0428 252 763 or email jstephens@berendsen.net.au

Northern MND Support Group

This is an informal catch-up style group, where members come together to talk all things MND and Kennedy's Disease, as well as share experiences and helpful tips. The group are once again catching up in person, which will coincide with the MND Clinic dates.

When: At 1:30pm on Wednesday 15th November and Wednesday 20th December

Where: Selah Café, Door of Hope, Glen Dhu Street, South Launceston

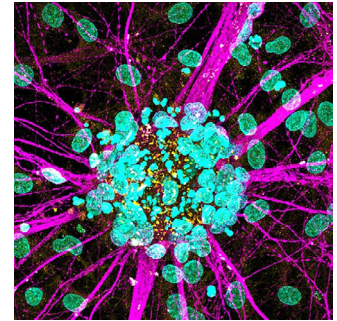
Contact: Emma Forsyth on 0456 182 551 or email eforsyth@mnd.org.au



MND RESEARCH

How research and the medical system develop medicines for MND in Australia

By Dr Gethin Thomas, MND Australia



Clinical trial of SPG302

Recent media stories have highlighted a new clinical trial currently underway in Melbourne. The trial, for a drug called SPG302, is being run on behalf of Spinogenix, the US company which makes the drug, by Nucleus Network.

SPG302 has been developed to target the synapse in nerve cells. The synapse is the site of contact between two nerve cells, or the nerve cell and muscle, that enables communication via chemical signals, and is key for the brain control of muscles for movement. One major aspect of MND is the loss of these synapses.

This drug has been designed to increase the number of synapses in nerve cells. The trial is a Phase 1 trial, which is the first step in testing whether a drug can develop into a viable treatment.

It will initially test how the drug behaves in healthy volunteers, to check for any toxic effects and how long it lasts in the body.

Early next year, there will also be a small part of this study which will test the drug in MND patients, but only for a very short period (4 weeks) to make sure that the drug behaves in the same way in patients as it does in healthy volunteers, and to measure any changes in patients that may occur.

It should be noted that, although it is exciting to have new drugs being tested, this is at a very early stage, and it will be a number of years before this drug, if successful, might make it into the clinic for patients.

According to research, the medicine Riluzole can prolong life with motor neurone disease (MND).¹ Riluzole is the only medicine for treatment of the disease available in Australia.²

By contrast, people living with MND in the United States of America (USA) can access three medicines: Riluzole, Edaravone and Relyvrio, and, just this month, Qalsody (previously Tofersen) which can all slow the progress of MND.^{3,4,5} But currently, Australians cannot access Qalsody, Edaravone and Relyvrio through our medical system.

Why don't people living with MND in Australia have access to medicines like Edaravone? And how can scientists develop medicines sooner?

We have looked at what helps a medicine progress from scientific experiments to doctors being able to prescribe it for MND in Australia. With better access to medicines, more people with MND can have greater choice over living with such a terrible disease.

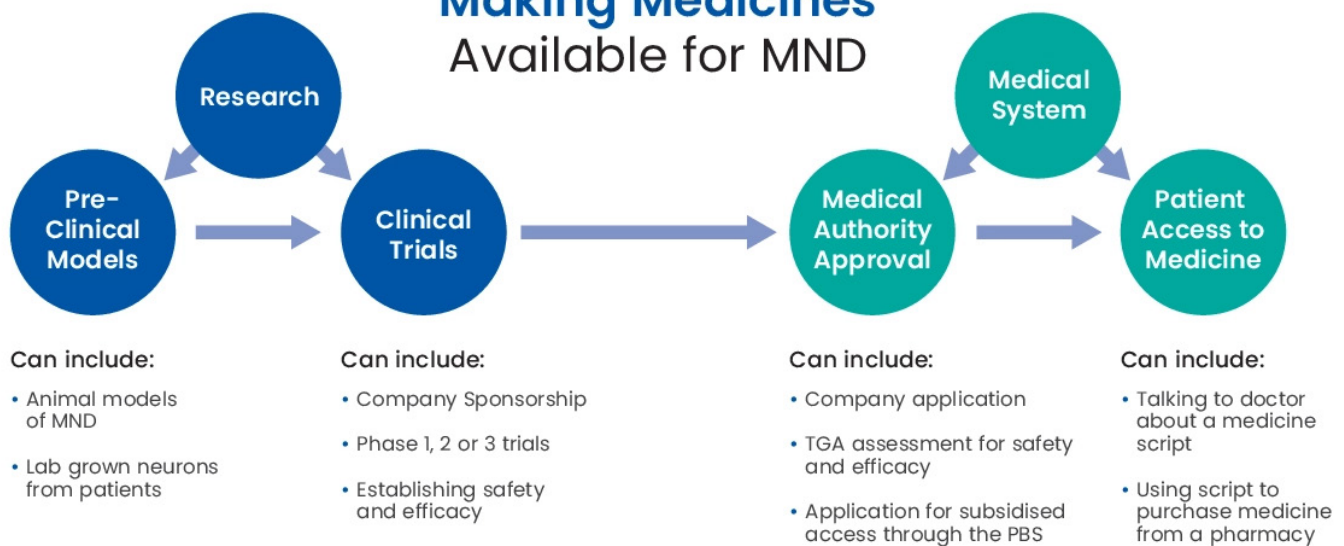
How do we find a new medicine?

Research is fundamental for developing new medicines and treatments for MND or any other disease or condition. Research is where a medicine for MND begins.

Scientists must rigorously test any potential treatment before its use by patients. First steps in testing often involve seeing if the new treatment works in "pre-clinical models". Models include lab grown neurons from patients and animal models of MND. If everything looks good after these early steps (also known as pre-clinical testing) the organisation developing the treatment may consider it for clinical trial.

Clinical trials are major research projects.⁶ Therefore, starting a clinical trial for a potential treatment requires approval. The organisation developing a treatment (usually a pharmaceutical or biotechnology company) is termed

Making Medicines Available for MND



the “sponsor”, and decides if they think the potential treatment is promising enough to take to trial.

They then apply for approval to the regulatory authorities in the country or countries where they want to run the trial. In Australia, for example, it is the Therapeutic Goods Administration (TGA). In the US, it is the Federal Drug Agency (FDA).

Clinical trials consist of phases. Usually, in Phase 1 or Phase 1/2 of a trial, testing determines treatment safety and that it won't cause harm. Scientists may also study what medicine dose might be best (the exact amount taken at one time) as part of the trial.

Phase 1 of a trial is usually very small. The trial will not involve sufficiently high numbers of participants to provide information on the effectiveness of a treatment. In fact, scientists often run Phase 1 trials on healthy volunteers rather than actual patients. A Phase 2 trial begins after Phase 1 research shows a medicine is safe.

Phase 2 trials mainly aim to finalise the best plan for medicine doses. Trials can also provide some provisional information on the potential effectiveness of a treatment. Scientists often design Phase 2/3 trials to help in starting to understand treatment effectiveness. Phase 2 trials are larger than Phase 1, especially if they are Phase 2/3, as they gather more data.

It is worth noting that it is not just the decision of the sponsor in determining if a treatment progresses to a clinical trial and through all its stages. At each stage, data must be presented to the local regulatory authority to show whether the treatment merits progression through the clinical trial pathway.

Phase 3 trials are the final phase of the clinical trial pathway. This trial is the last stage before a sponsor seeks approval to make the treatment available to the public. Scientists specifically design Phase 3 trials to show whether a drug works. The trials need to be much larger than Phase 1 or 2 trials. The reason for the larger size is to establish statistical certainty for definitively saying whether a medicine works or not.

Phase 3 of trials are very expensive (often in the 100s of millions of dollars). In addition to the cost, the trials can take well over 12 months. Trials must be set-up, participants recruited for treatment, and analyses conducted. The time and cost are necessary to try and make sure that medicines actually work and are safe to use.

From research to pharmacy shelves: making a new treatment available to patients

Once a treatment has been through the clinical trial pipeline and found to be safe and effective, what happens next? How do patients access medicines?

Unfortunately, it is not an automatic progression from trial success to availability to patients. Often, at first glance, the outcomes of clinical trials may appear to be positive. But on closer analysis, trial results may indicate that the benefits were not as great as first thought. Conversely, there may have been confounding reasons that resulted in a trial outcome appearing worse than it really was.

To best determine the quality of trial outcomes, a local regulatory authority requires the trial sponsor to submit an application for medicine approval. The application allows the authority to objectively and independently review the data.

The authority make their own decision on the benefit of a particular treatment. Such assessment will involve a number of experts in clinical trials, statistics, and the disease area in question.

Assessments are very involved considerations, and can often take 6 months or more. As well as considering the effectiveness and safety profile of the treatment, the process considers how medicines are scheduled for prescribing by doctors, and whether they are listed on the Pharmaceutical Benefits Scheme (PBS), which helps to make medicines available at a lower cost.

Currently, the TGA grants initial approval based purely on whether the evidence shows a treatment is safe and effective.⁷ The TGA offers very limited opportunity for input into their approval decisions by advocacy organisations such as MND Australia.

The organisation sponsoring development of the medicine completes the application. Some organisations keep applications confidential, making it hard to even know an application is underway. However, in the MND community, which tends to work collaboratively whenever possible, it would be unlikely for an application to proceed without the MND community knowing.

What can help make more medicines available for MND

Clinical trials and regulatory authority approval can take quite a long time. But there are ways to make more MND medicines available sooner.

Ongoing investment in the work of MND scientists in Australia and across the world is critical. Research is best for finding safe and effective medicines that benefit people with MND. Donations, fundraising campaigns and other opportunities to support MND research remain vital for research into medicines.

Beyond research, working with organisations sponsoring medicines can influence the process of TGA approval in Australia. MND Australia and others can, and do, work with sponsors when they are preparing their submissions. MND Australia provides data and brings the experiences of those with lived experience to the table. Sharing data and experience helps to increase the likelihood of approval for a submission.

Working to persuade sponsors to come to Australia, to help them understand our system, and to offer support helps too. Some companies developing new treatments do not have deep

resources, making targeting multiple countries simultaneously for approval difficult.

This is the case for Amylyx, the company which developed Relyvrio, which was recently approved for MND treatment in Canada and USA. They need to build up their manufacturing capacity to service the USA and Canada, which will then establish a steady income stream so that they can then look to expand their reach. MND Australia is already scoping how we can best support companies which are considering applying for medicine approval with the TGA.

In addition to medicine approval, MND Australia and other organisations can help with making medicines more affordable. The application process for subsidised treatment through the PBS does provide an opportunity for organisations to lobby for change.

Lobbying normally takes the form of an organisation completing a written submission about the need for a treatment and its benefits. Organisations submit applications to the TGA review committee. The testimonials of people with, or affected by, MND, can support submissions, because they help to share the experiences of daily life from living with a terrible disease like MND, and why an affordable medicine is so important.

MND Australia are also lobbying for greater involvement of people with MND in TGA decision making more broadly. MND Australia is participating in the review of Australia's Health Technology Assessment. The review seeks to help reduce the wait times for patients wanting to access new and innovative medicines.

Stay informed

Creating a world without MND, through the search for better medicines, and a cure, is a complicated and difficult challenge. Work is underway though, and there are many opportunities to improve access to medicines and life with MND.

It can help to stay informed about what the TGA is considering for MND medicines. Being aware of medicines already available, including Riluzole and medicines for pain relief, is important.

Doctors, neurologists and other members of the healthcare team for a person living with MND can advise on how to access and use Riluzole and other medicines.

Sources

1. A. Chio, L. Mazzini and G. Mora. 2020. "Disease-modifying therapies in amyotrophic lateral sclerosis." *Neuropharmacology*. 167:107986. doi: 10.1016/j.neuropharm.2020.107986.
2. T. Dharmadasa et al. 2017. "Motor neurone disease: progress and challenges." *Med J Aust*. 206(8): 357-362. doi: 10.5694/mja16.01063.
3. X. Xu et al. 2021. "A perspective on therapies for amyotrophic lateral sclerosis: can disease progression be curbed?" *Transl. Neurodegener.* 10(1): 29. 10.1186/s40035-021-00250-5.
4. Y. Sun, X. Li, and R. Bedlack. 2023. "An evaluation of the combination of sodium phenylbutyrate and taurursodiol for the treatment of amyotrophic lateral sclerosis." *Expert. Rev. Neurother.* 23(1):1-7. doi: 10.1080/14737175.2023.2174018.
5. T. M. Miller et al. 2022. "Trial of Antisense Oligonucleotide Tofersen for SOD1 ALS." *N Engl J Med*. 387(12):1099-1110. doi: 10.1056/NEJMoa2204705.
6. Australian Government. 2023. "Why conduct a clinical trial in Australia." *Australian Clinical Trials*. Apr. 14: <https://www.australianclinicaltrials.gov.au/why-conduct-clinical-trial-australia> (accessed Jan. 05, 2023).
7. S. J. Ayre, C. Bonner, D.M. Muscat, A.G. Dunn, E. Harrison, J. Dalmazzo, D. Mouwad, P. Aslani, H.L. Shepherd, K. J. McCaffery. 2023. *Automated Health Literacy Assessments of Written Health Information: Development of the SHeLL (Sydney Health Literacy Lab) Health Literacy Editor v1*. *JMIR Formative Research*. e40645. DOI: 10.2196/40645
7. Therapeutic Goods Administration. 2021. "Prescription medicines registration process." Therapeutic Goods Administration, Aug. 12: <https://www.tga.gov.au/how-we-regulate/supply-therapeutic-good/supply-prescription-medicine/application-process/prescription-medicines-registration-process>.

FUNdraise for MND Tasmania

Are you interested in becoming a community fundraiser for MND Tasmania? You could host an event, get active, set a challenge, celebrate an occasion, have a bake sale... ideas are only limited by your imagination!

When you sign up to fundraise for us, you'll receive your own online fundraising page to help spread the word about your activity and to receive donations. Get in touch with us by emailing info@mndatas.asn.au



PERSONAL STORY

Amy's Story

By Amy Lynch

I always dreamed of being a mother. I'm naturally very maternal and love spending time with kids and animals. So my husband, Simon and I, were overjoyed when we welcomed our first baby girl, Hannah, in December 2020. 17 months later, we welcomed our second baby girl, Georgia.

Motherhood is never quite what you expect it to be, and often it can be challenging. But for our little family, things are tougher than for most.

At only 27 years old, when Georgia was just 10 weeks old, I was diagnosed with motor neurone disease. Being so young it's quite rare, but not unheard of.

MND is a prison. One of the hardest things is that I feel perfectly normal but then I go to take a step and my leg doesn't respond. Or I go to move my hand and it doesn't go where I've told it to. **It's very frustrating to be trapped in my own body.** My mind is fully active, but my hands, legs and tongue don't do what I ask them to do.

I first noticed something wasn't right when I was pregnant with Georgia. I started walking a bit funny but, being pregnant, I didn't think much of it. I was still able to do everything - potter around our farm, chase after a 1 year old, renovate our house.

A few days after having Georgia, I started to have weakness in my hands, and when I left the hospital, I was walking a bit funny again and used the pram as a walker.

But then a couple of weeks later, I was playing around with Hannah and she pushed me over a couple of times. She was only 18 months old at the time so it was quite shocking that she was able to knock me off balance. That's when I went and saw our local GP. He sent me for every test under the sun to try and find out what was wrong.

He said: "Don't be alarmed. With the marvels of modern medicine, they can pretty much manage or treat anything. You'll have a normal life." Little did we know that I had one of the diseases that has no known treatment or cure.

When Trish, our MND Advisor/Support Coordinator called, it was a very sobering moment to realise that this is really what we're looking at. There's almost a bit of freedom when you don't know what's wrong with you yet, because there's a chance that there's nothing wrong. **You definitely never think it'll be what you end up with. MND really is the worst.**

Right from that very first phone call, MND Victoria's support has made a lot of difference. Trish helps us navigate the NDIS and gets us connected with all of the support services which is invaluable. Because, without that bit of guidance, we wouldn't know where to start.

Living on a farm in rural Victoria can sometimes make it tricky to get access to things, but Trish has been terrific in sourcing services and suppliers who are happy to travel to the area to support me. Especially because often a carer isn't just caring for me - they're helping me while I care for the kids.

One silver lining of my diagnosis is that we get to spend so much time together as a family. Simon primarily works from home so that he can be there to take care of Hannah, Georgia and me. We go on as many holidays as we can and travel around to see all of our family and friends. We do all the things we want to do, but we know our timeline to do these things is shorter than for most families.

I'm quite a caring person and always put my energy into making other people's lives better, so it's quite surreal that now I'm the one being cared for. We have a young family and the last thing we want is to not have that together.

Our life's just starting off, not finishing up. So the longer I can be part of that and be in the middle of it all instead of watching from the outside, the better. We're trying to keep life as normal as it can be and make lots of memories with the kids. And the support we receive from MND Victoria really helps us to achieve that.



Right from that very first phone call,
MND Victoria's support has made
a lot of difference.



Scan this QR code to
watch Amy's story



MND INFORMATION

Client Numbers

We are currently supporting 57 registered members:

North West Region Tasmania	9
North Region Tasmania	20
Southern Tasmania	28
TOTAL	57



**CURRENTLY
SUPPORTING
57
REGISTERED
MEMBERS**



**DELIVERED
1,338
HOURS OF
SERVICE
in the last quarter**

MND Tasmanian Board Members:

MND Tasmania has a volunteer Board and no paid staff.

President: Kate Todd

Senior Vice President: Tracey Dickson

Vice President & Public Officer: Chris Symonds

Secretary: Lucy Polizzi

Treasurer: Julie Driessen

Member Support: Elisa Howlett

Fundraising: Saga Hassinen

Ordinary Directors: Steve Isaac

MND Advisors and NDIS Support Co-ordinators

Northern Tasmania: Emma Forsyth (Mon - Thur):

p: 0456 182 551 or e: eforsyth@mnd.org.au

Southern Tasmania: Jenny Fuller (Tue, Wed, Fri):

p: 0412 599 365 or e: jfuller@mnd.org.au

Team leader: Kim Hamilton (Tue - Fri):

p: 0422 149 445 or e: khamilton@mnd.org.au

FREECALL 1800 777 175

This number is at the MND Victoria office (Monday - Friday, 9 am - 5 pm). To assist the volunteer receptionist, please say that you are calling MND Tasmania. If you wish to speak to a Board member or an MND Advisor, you will be asked for your contact details and your call will be returned by that person as soon as possible.

*Right: Emma Forsyth
Below: Jenny Fuller*



We acknowledge Aboriginal and Torres Strait Islander peoples as the Traditional Custodians of the lands in which we live, work, and learn. We recognise and respect the enduring relationship they have with their lands and waters, and we pay our respects to Elders past, present, and emerging.

MND TASMANIA

PO Box 379, Sandy Bay,
TAS 7006 Australia

Freecall: 1800 777 175

Charity ABN: 21877144292

✉ info@mndatas.asn.au
🖱 www.mndatas.asn.au
f www.facebook.com/MNDTas